4164-01-P

DEPARTMENT OF HEALTH AND HUMAN SERVICES

Food and Drug Administration

[Docket No. FDA-2018-N-0663]

Tissue Agnostic Therapies in Oncology: Regulatory Considerations for Orphan Drug Designation; Public Workshop; Request for Comments

AGENCY: Food and Drug Administration, HHS.

ACTION: Notice of public workshop; request for comments.

SUMMARY: The Food and Drug Administration (FDA or the Agency) is announcing the following public workshop entitled "Tissue Agnostic Therapies in Oncology: Regulatory Considerations for Orphan Drug Designation." The purpose of the public workshop is to discuss factors FDA should consider when evaluating drugs for orphan designation that treat a tissue agnostic disease or condition in oncology, and additional factors related to orphan exclusivity FDA should consider when approving a product with a tissue agnostic indication.

DATES: The public workshop will be held on May 9, 2018, from 9 a.m. to 5 p.m. The public workshop may be extended or may end early depending on the level of public participation. Submit either electronic or written comments on this public workshop by June 8, 2018. See the SUPPLEMENTARY INFORMATION section for registration date and information.

ADDRESSES: The public workshop will be held at the FDA White Oak Campus, 10903 New Hampshire Ave., Building 31 Conference Center, the Great Room (Rm. 1503, Section A), Silver Spring, MD 20993-0002. Entrance for the public workshop participants (non-FDA employees) is through Building 1, where routine security check procedures will be performed. For parking and security information, please refer to

https://www.fda.gov/AboutFDA/WorkingatFDA/BuildingsandFacilities/WhiteOakCampusInformation/ucm241740.htm.

You may submit comments as follows. Please note that late, untimely filed comments will not be considered. Electronic comments must be submitted on or before June 8, 2018. The https://www.regulations.gov electronic filing system will accept comments until midnight Eastern Time at the end of June 8, 2018. Comments received by mail/hand delivery/courier (for written/paper submissions) will be considered timely if they are postmarked or the delivery service acceptance receipt is on or before that date.

Electronic Submissions

Submit electronic comments in the following way:

- Federal eRulemaking Portal: https://www.regulations.gov. Follow the instructions for submitting comments. Comments submitted electronically, including attachments, to https://www.regulations.gov will be posted to the docket unchanged. Because your comment will be made public, you are solely responsible for ensuring that your comment does not include any confidential information that you or a third party may not wish to be posted, such as medical information, your or anyone else's Social Security number, or confidential business information, such as a manufacturing process. Please note that if you include your name, contact information, or other information that identifies you in the body of your comments, that information will be posted on https://www.regulations.gov.
- If you want to submit a comment with confidential information that you do not wish to be made available to the public, submit the comment as a written/paper submission and in the manner detailed (see "Written/Paper Submissions" and "Instructions").

Written/Paper Submissions

Submit written/paper submissions as follows:

- Mail/Hand delivery/Courier (for written/paper submissions): Dockets Management Staff (HFA-305), Food and Drug Administration, 5630 Fishers Lane, Rm. 1061, Rockville, MD 20852.
- For written/paper comments submitted to the Dockets Management Staff, FDA will post your comment, as well as any attachments, except for information submitted, marked and identified, as confidential, if submitted as detailed in "Instructions."

Instructions: All submissions received must include the Docket No. FDA-2018-N-0663 for "Tissue Agnostic Therapies in Oncology: Regulatory Considerations for Orphan Drug Designation; Public Workshop; Request for Comments." Received comments, those filed in a timely manner (see ADDRESSES), will be placed in the docket and, except for those submitted as "Confidential Submissions," publicly viewable at https://www.regulations.gov or at the Dockets Management Staff between 9 a.m. and 4 p.m., Monday through Friday.

- Confidential Submissions--To submit a comment with confidential information that you do not wish to be made publicly available, submit your comments only as a written/paper submission. You should submit two copies total. One copy will include the information you claim to be confidential with a heading or cover note that states "THIS DOCUMENT CONTAINS CONFIDENTIAL INFORMATION." The Agency will review this copy, including the claimed confidential information, in its consideration of comments. The second copy, which will have the claimed confidential information redacted/blacked out, will be available for public viewing and posted on https://www.regulations.gov. Submit both copies to the Dockets

Management Staff. If you do not wish your name and contact information to be made publicly available, you can provide this information on the cover sheet and not in the body of your comments and you must identify this information as "confidential." Any information marked as "confidential" will not be disclosed except in accordance with 21 CFR 10.20 and other applicable disclosure law. For more information about FDA's posting of comments to public dockets, see 80 FR 56469, September 18, 2015, or access the information at: https://www.gpo.gov/fdsys/pkg/FR-2015-09-18/pdf/2015-23389.pdf.

Docket: For access to the docket to read background documents or the electronic and written/paper comments received, go to https://www.regulations.gov and insert the docket number, found in brackets in the heading of this document, into the "Search" box and follow the prompts and/or go to the Dockets Management Staff, 5630 Fishers Lane, Rm. 1061, Rockville, MD 20852.

FOR FURTHER INFORMATION CONTACT: Nicole Wolanski, Office of Orphan Products Development, Food and Drug Administration, 10903 New Hampshire Ave., Bldg. 32, Rm. 5210, Silver Spring, MD 20933, 301-796-6570, OOPDOrphanEvents@fda.hhs.gov.

SUPPLEMENTARY INFORMATION:

I. Background

The combination of government incentives, scientific advances, and the promise of commercial opportunity has fueled extraordinary investment in orphan drugs. Since the Orphan Drug Act was first passed in 1983, over 650 rare disease indications for drugs and biologics have been developed and approved for marketing. In fact, rare disease drug approvals have accounted

for approximately 40 percent of the new molecular entities and therapeutic biologic products in the Center for Drug Evaluation and Research for the last several years.

Not only have we seen tremendous growth in the development of products for rare diseases, but the very landscape of rare disease product development is changing, with an increase in the development of targeted therapies, more interest in the development of biologics (including gene therapies), and tremendous growth in the oncology space. For example, in 2017 alone, FDA granted its first tissue agnostic approval (pembrolizumab for patients with unresectable or metastatic, microsatellite instability-high (MSI-H) or mismatch repair deficient (dMMR) solid tumors) and first tissue agnostic orphan drug designations (larotrectinib and entrectinib, each for the treatment of solid tumors with NTRK-fusion proteins). FDA also approved the first cell-based gene therapy, KYMRIAH, for use in treating a rare pediatric cancer.

As advancements in genomics and precision medicine continue, FDA has been taking these new developments into account as it considers what constitutes a "disease or condition." For example, one question that has already arisen in oncology is whether a disease should be defined in a tissue/organ-specific or a tissue agnostic manner. Because the continued development of targeted therapies for molecularly defined groups has the potential to alter the landscape of orphan drug development, FDA is holding the public workshop to obtain input on the complex scientific and regulatory issues surrounding molecularly targeted drugs and biologics in oncology and the appropriate application of orphan drug incentives in that paradigm. This discussion will inform how the Agency can incorporate the latest science and drug development trends into the implementation of the Orphan Drug Act, all while continuing to reflect the goals intended by Congress.

II. Topics for Discussion at the Public Workshop

This public workshop will consist of both presentations and interactive panel discussions. The presentations will provide information to outline the goals of the workshop and help promote interactive discussions. Following the presentations, there will be a moderated discussion where speakers and additional panelists will be asked to provide their individual perspectives. The presentations and discussions will focus on several related topics. Topics will involve discussion of and seek input on factors FDA should consider when evaluating drugs for orphan designation that treat a tissue agnostic disease or condition in oncology and additional factors related to orphan exclusivity to consider when approving a product with a tissue agnostic indication. A detailed agenda will be posted on the following website in advance of the workshop: https://www.fda.gov/NewsEvents/MeetingsConferencesWorkshops/ucm592778.htm.

III. Participating in the Public Workshop

Registration: To register for the public workshop, please visit the following website by April 25, 2018: https://www.fda.gov/NewsEvents/MeetingsConferencesWorkshops/ucm592778.htm. Please provide complete contact information for each attendee, including name, title, affiliation, address, email, and telephone.

Registration is free and based on space availability, with priority given to early registrants. Persons interested in attending this public workshop must register by April 25, 2018, 5 p.m. Eastern Time. Early registration is recommended because seating is limited; therefore, FDA may limit the number of participants from each organization. Registrants will receive confirmation when their registration has been received. If time and space permit, onsite registration on the day of the public workshop will be provided beginning an hour prior to the start of the meeting.

If you need special accommodations due to a disability, please contact Nicole Wolanski, at 301-796-6570, or OOPDOrphanEvents@fda.hhs.gov no later than April 25, 2018.

An agenda for the workshop and any other background materials will be made available 5 days before the workshop at https://www.fda.gov/NewsEvents/MeetingsConferencesWorkshops/ucm592778.htm.

Streaming Webcast of the Public Workshop: For those unable to attend in person, FDA will provide a live webcast of the workshop. To register for the streaming webcast of the public workshop, please visit the following website by May 8, 2018: https://www.fda.gov/NewsEvents/MeetingsConferencesWorkshops/ucm592778.htm.

If you have never attended a Connect Pro event before, test your connection at https://collaboration.fda.gov/common/help/en/support/meeting_test.htm. To get a quick overview of the Connect Pro program, visit https://www.adobe.com/go/connectpro_overview. FDA has verified the website addresses in this document, as of the date this document publishes in the *Federal Register*, but websites are subject to change over time.

Transcripts: Please be advised that as soon as a transcript of the public workshop is available, it will be accessible at https://www.regulations.gov. It may be viewed at the Dockets Management Staff (see ADDRESSES). A link to the transcript will also be available on the internet at https://www.fda.gov/NewsEvents/MeetingsConferencesWorkshops/ucm592778.htm.

Dated: February 22, 2018.

Leslie Kux,

Associate Commissioner for Policy.

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